International Journal of Cell Science and Biotechnology
E-ISSN 2320-7574

Available at Available at https://ijcsb.evegenis.org/

Peer Reviewed Article

Applications of stem cells in human degenerative diseases

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Received 10 April 2020, Accepted 20 May 2020, Available online 25 May 2020, **Vol.9 (2020)**

Abstract

Stem cells hold immense potential for the treatment of various human diseases due to their unique ability to differentiate into different cell types and their capacity for self-renewal. The applications of stem cells in regenerative medicine have opened new avenues for the treatment of degenerative diseases, such as Parkinson's disease, Alzheimer's disease, and spinal cord injuries, by replacing damaged or lost cells with healthy ones derived from stem cells. In cardiovascular diseases, stem cells are being explored for their potential to repair and regenerate damaged heart tissue, offering hope for patients with heart failure and myocardial infarction. In addition to regenerative medicine, stem cells are being utilized in the field of cancer research, where they play a crucial role in understanding tumor biology, developing new anticancer therapies, and potentially eradicating cancer stem cells to prevent tumor recurrence. Hematopoietic stem cell transplantation is already a well-established treatment for blood disorders like leukemia and lymphoma, demonstrating the life-saving potential of stem cell therapy. Furthermore, stem cells are being investigated for their use in treating diabetes by generating insulin-producing cells, and in genetic disorders through gene therapy approaches. Despite the promising advancements, challenges such as ethical concerns, immune rejection, and the risk of tumorigenesis must be addressed before stem cell therapies can be widely adopted. Nonetheless, the ongoing research and clinical trials continue to pave the way for innovative and personalized treatments for a variety of human diseases, underscoring the transformative potential of stem cells in modern medicine.

Keywords: *Stem cells, Human diseases, Pluripotency, Differentiation.*

1. Introduction

Stem cells have emerged as a cornerstone in the field of regenerative medicine due to their unique ability to self-renew and differentiate into various specialized cell types. These pluripotent or multipotent cells offer unprecedented potential for treating a wide range of human diseases, including those that are currently incurable or difficult to manage with conventional therapies. The concept of using stem cells to repair or replace damaged tissues has revolutionized the approach to treating degenerative diseases, genetic disorders, and even certain forms of cancer.

One of the most well-established applications of stem cells is in the treatment of hematological disorders through hematopoietic stem cell transplantation (HSCT). This approach has been successfully used for decades to treat patients with leukemia, lymphoma, and other blood-related conditions by replenishing the patient's blood cells after high-dose chemotherapy or radiation therapy (Thomas *et al.*, 1957).

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DOI: https://doi.org/10.14741/ijcsb/v.9.1.1

The success of HSCT has laid the groundwork for exploring the therapeutic potential of other types of stem cells, such as embryonic stem cells (ESCs), induced pluripotent stem cells (iPSCs), and mesenchymal stem cells (MSCs), in a broader range of diseases.

In the context of neurodegenerative diseases, stem cells are being investigated for their ability to replace lost neurons and restore function in conditions such as Parkinson's disease, Alzheimer's disease, and amyotrophic lateral sclerosis (ALS). For example, recent studies have shown promising results in using iPSCs to generate dopaminergic neurons for transplantation into patients with Parkinson's disease, offering potential symptom relief and disease modification (Takahashi & Yamanaka, 2006). Similarly, stem cell-derived neural progenitors are being explored for their ability to regenerate damaged brain tissue in Alzheimer's disease, though clinical applications remain in the early stages of development (Selkoe & Hardy, 2016).

Cardiovascular diseases, another leading cause of mortality worldwide, have also seen advancements through stem cell research. Cardiomyocytes derived from stem cells are being tested for their potential to repair heart tissue damaged by myocardial infarction,

aiming to improve cardiac function and reduce the burden of heart failure (Laflamme *et al.*, 2007). Moreover, stem cells are being used to develop bioengineered heart tissues and patches, which could provide structural support and functional recovery in damaged hearts (Eschenhagen *et al.*, 2017).

Despite these advancements, several challenges remain in translating stem cell therapies from bench to bedside. Ethical concerns, particularly surrounding the use of ESCs, continue to provoke debate, while issues related to immune rejection, tumorigenesis, and the long-term safety of stem cell therapies need to be addressed through rigorous research and clinical trials (Lo & Parham, 2009). Nonetheless, the potential applications of stem cells in treating human diseases remain vast, offering hope for the development of innovative and personalized therapeutic strategies in the future.

2. Human Degenerative Disorders

Human degenerative disorders, such as Parkinson's disease, Alzheimer's disease, and amyotrophic lateral sclerosis (ALS), represent some of the most challenging conditions in modern medicine due to their progressive nature and lack of effective treatments. These disorders are marked by the gradual loss of neurons and the accumulation of pathological proteins, leading to irreversible damage to the nervous system and the decline of cognitive and motor functions. Current therapeutic approaches are largely symptomatic and do not halt the progression of the disease, creating an urgent need for innovative treatment strategies. Stem cell therapy has emerged as a promising approach for addressing these challenges by offering the potential to replace lost neurons, modulate neuroinflammation, and restore neural networks. Recent advances in stem cell research have demonstrated the potential of induced pluripotent stem cells (iPSCs) and mesenchymal stem cells (MSCs) in generating dopaminergic neurons for Parkinson's disease, which could significantly improve motor function and quality of life for patients (Parmar *et al.*, 2020). Similarly, in Alzheimer's disease, stem cell-derived neural progenitors are being explored for their capacity to integrate into damaged brain regions and potentially slow down or reverse cognitive decline. Moreover, the application of stem cells in ALS has shown promise in protecting motor neurons from degeneration and extending patient survival). While these therapies are still in experimental stages, the growing body of evidence suggests that stem cell-based interventions could revolutionize the treatment of neurodegenerative disorders, offering hope for slowing disease progression and improving patient outcomes.

3. Role of stem cells in human degenerative disorders

Stem cells have emerged as a pivotal element in the ongoing quest to treat and manage human degenerative disorders, which are often characterized by the progressive loss of specific cell types and the deterioration of tissues and organs. These disorders, including neurodegenerative diseases such as Parkinson's disease, Alzheimer's disease, and amyotrophic lateral sclerosis (ALS), as well as degenerative conditions affecting other systems like osteoarthritis and heart failure, present significant challenges due to their chronic nature and limited treatment options. The role of stem cells in addressing these challenges is multi-faceted, encompassing cell replacement therapies, disease modeling, drug testing, and the potential for understanding the mechanisms of degeneration at a cellular level.

One of the most promising applications of stem cells in human degenerative disorders is their potential for cell replacement therapy. In conditions like Parkinson's disease, where dopaminergic neurons are progressively lost, stem cells offer the possibility of generating new, functional neurons that can be transplanted into patients to restore lost functions. Recent studies using induced pluripotent stem cells (iPSCs) have shown that these cells can be differentiated into dopaminergic neurons, which, when transplanted into animal models of Parkinson's disease, have resulted in improved motor function and a reduction in disease symptoms (Takahashi, 2020). These findings have paved the way for ongoing clinical trials aimed at testing the safety and efficacy of iPSC-derived dopaminergic neurons in human patients, representing a significant step toward a potential cure for Parkinson's disease.

In Alzheimer's disease, the role of stem cells is equally promising, although more complex due to the widespread and multifactorial nature of neuronal loss in the disease. Researchers are exploring the use of neural stem cells (NSCs) to replace damaged neurons and support the regeneration of neural networks in the brain. In addition to their potential for replacing lost neurons, NSCs have been shown to secrete neurotrophic factors that support the survival of existing neurons and modulate neuroinflammation, which is a key feature of Alzheimer's pathology (Blurton-Jones *et al.*, 2019). The ability of stem cells to influence the brain's microenvironment and potentially alter the disease course makes them a valuable tool not only for cell replacement but also for modifying disease progression. Current research efforts are focused on optimizing the delivery methods and understanding the long-term effects of stem cell therapies in Alzheimer's disease, with some studies already showing promising results in animal models.

Amyotrophic lateral sclerosis (ALS) is another devastating neurodegenerative disorder where stem cells are being investigated as a potential treatment. The disease is characterized by the degeneration of motor neurons, leading to progressive muscle weakness and eventual paralysis. Stem cells offer a potential strategy for replacing lost motor neurons and protecting remaining neurons from further degeneration. Mesenchymal stem cells (MSCs) have been particularly studied for their neuroprotective properties, as they can

secrete factors that reduce inflammation and oxidative stress, both of which are implicated in ALS progression (Satti *et al.*, 2016; Mazzini *et al.*, 2019). Additionally, iPSCs derived from ALS patients are being used to model the disease in vitro, allowing researchers to study the mechanisms of motor neuron degeneration and screen potential therapeutic compounds (Birger *et al.*, 2019). These patient-specific iPSCs provide a powerful platform for personalized medicine, where treatments can be tailored to the individual's unique genetic and cellular makeup.

Beyond neurodegenerative disorders, stem cells are also being explored for their role in treating degenerative conditions in other systems. For instance, in osteoarthritis, where the degradation of cartilage leads to joint pain and dysfunction, stem cells have shown potential in regenerating cartilage tissue. Studies have demonstrated that MSCs can differentiate into chondrocytes, the cells responsible for producing cartilage, and promote the repair of damaged joints (McIntyre *et al.*, 2018. Clinical trials have shown that intra-articular injections of MSCs in patients with osteoarthritis can lead to significant improvements in pain and joint function, suggesting that stem cell therapy could become a viable alternative to joint replacement surgery (Jo *et al.*, 2017). Similarly, in cardiovascular diseases, stem cells are being used to regenerate heart tissue damaged by myocardial infarction. Cardiac stem cells (CSCs) and iPSC-derived cardiomyocytes have shown promise in preclinical studies for repairing damaged heart tissue and improving heart function (Menasché *et al.*, 2018; Yoshida & Yamanaka, 2017).

The use of stem cells in these applications is not without challenges. One of the primary concerns is the potential for tumorigenesis, particularly when using pluripotent stem cells like iPSCs, which have the ability to form teratomas if not properly differentiated before transplantation. Another significant challenge is the risk of immune rejection, especially in allogeneic stem cell transplants, where the patient's immune system may recognize the transplanted cells as foreign and mount an immune response. Strategies to overcome these challenges include the use of autologous stem cells, where the patient's own cells are reprogrammed and differentiated into the desired cell type, thereby minimizing the risk of rejection. Additionally, ongoing research into immunomodulatory therapies and the development of universal donor cells that can evade immune detection is expected to improve the safety and efficacy of stem cell-based treatments (Porteus, 2019).

Despite these challenges, the potential benefits of stem cell therapy in treating degenerative disorders are immense. Stem cells not only offer the possibility of replacing lost or damaged cells but also provide a platform for understanding the underlying mechanisms of disease and for testing new drugs and therapies. The advent of gene editing technologies like CRISPR has further expanded the potential of stem cells by allowing precise modifications to be made to the genome, enabling the correction of genetic defects that cause degenerative disorders (Schwartz & Wang, 2020). This synergy between stem cell biology and gene editing holds great promise for developing curative therapies for a wide range of degenerative conditions.

4. Future Directions

The future of stem cell therapy in treating human degenerative disorders is poised for remarkable advancements, driven by ongoing research and technological innovations. One promising direction is the integration of stem cell therapy with gene editing technologies like CRISPR-Cas9, enabling precise correction of genetic defects responsible for various degenerative diseases and enhancing the functionality and safety of transplanted cells. Advances in biomaterials and tissue engineering are also expected to improve stem cell delivery and integration, allowing for the development of bioengineered tissues and organs tailored to individual patient needs. Furthermore, the emergence of personalized medicine approaches will facilitate the creation of patient-specific stem cell lines, reducing the risk of immune rejection and improving therapeutic outcomes. Enhanced understanding of stem cell biology and the microenvironmental cues that govern cell differentiation and regeneration will contribute to more effective and targeted treatments. Additionally, large-scale, well-designed clinical trials are necessary to establish standardized protocols and assess long-term safety and efficacy across diverse patient populations. Ethical and regulatory frameworks will continue to evolve, ensuring responsible and equitable access to these cutting-edge therapies. Collaborations across multidisciplinary fields, including biotechnology, nanotechnology, and computational biology, will further accelerate progress and open new avenues for innovation. Ultimately, these advancements hold the promise of transforming the treatment landscape for degenerative disorders, offering improved quality of life and potentially curative solutions for patients worldwide.

Conclusions

Stem cells have revolutionized the approach to treating human degenerative disorders by offering the potential for not only alleviating symptoms but also addressing the underlying causes of these diseases. The ability of stem cells to differentiate into various cell types and their capacity for self-renewal make them invaluable tools in regenerative medicine. From neurodegenerative conditions like Parkinson's disease, Alzheimer's disease, and amyotrophic lateral sclerosis (ALS) to degenerative disorders affecting other systems such as osteoarthritis and heart disease, stem cell-based therapies hold promise for replacing damaged cells, modulating disease progression, and restoring function. Significant advancements have been made in the development of induced pluripotent stem cells (iPSCs)

and mesenchymal stem cells (MSCs) for cell replacement therapies, with ongoing clinical trials exploring their efficacy and safety in humans. Additionally, the use of stem cells in disease modeling has provided new insights into the mechanisms of degeneration, allowing for the development of more targeted and effective treatments. However, challenges remain, including the risks of tumorigenesis, immune rejection, and ensuring the long-term safety of these therapies.

Despite these obstacles, the progress in stem cell research continues to offer hope for the development of innovative treatments that could transform the management of degenerative disorders. With further research and refinement, stem cell therapies could move from experimental stages to becoming a standard part of medical practice, offering new possibilities for millions of patients worldwide. The integration of stem cell therapy with emerging technologies like gene editing holds the potential to not only treat but possibly cure many degenerative diseases, marking a new era in personalized and regenerative medicine.

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